
Optogenetic therapy for treating retinitis pigmentosa and other inherited retinal diseases

Grant Award Details

Optogenetic therapy for treating retinitis pigmentosa and other inherited retinal diseases

Grant Type: Therapeutic Translational Research Projects

Grant Number: TRAN1-13342

Investigator:

Name:	Paul Bresge
Institution:	Ray Therapeutics, INC
Type:	PI

Disease Focus: Retinitis Pigmentosa, Vision Loss

Human Stem Cell Use: Somatic Cell

Award Value: \$3,999,553

Status: Pre-Active

Grant Application Details

Application Title: Optogenetic therapy for treating retinitis pigmentosa and other inherited retinal diseases

Public Abstract:**Translational Candidate**

Ray-001 is an AAV gene therapy delivering a light sensitive gene to treat patients with advanced Retinitis Pigmentosa.

Area of Impact

Retinitis pigmentosa (RP) is a genetic disease that causes retinal degeneration leading to near or complete blindness for most patients.

Mechanism of Action

Ray Therapeutics' therapy (Ray-001) delivers a potent transgene with high sensitivity to light incoming to the eye, and high dynamic range. Ray-001 has been shown to effectively treat the target retinal neurons. Light activation of the protein delivered by Ray-001 results in a signal being sent to the visual cortex of the brain.

Unmet Medical Need

Ray-001 will treat patients with advanced retinitis pigmentosa (RP) and other inherited retinal diseases (IRD) who currently have no other approved treatment.

Project Objective

pre-IND meeting

Major Proposed Activities

- Preclinical animal studies to further evaluate safety and efficacy
- Manufacturing process and analytical development
- Preparation for a pre-IND meeting

Statement of Benefit to California:

Retinitis pigmentosa (RP) is a progressively debilitating disease which leads to blindness. Of the approximate 10,000 patients living with RP in California, many have advanced disease, to the point of total loss of visual acuity. Most of these patients need to receive healthcare benefits, special living assistance, and suffer from loss of financial independence. Ray-001 represents a potential breakthrough treatment for a high unmet medical need for RP patients to improve their quality of life.

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